

CLAIMS:

1. A nucleic acid molecule comprising nucleic acid sequence encoding microtrophin under the control of regulatory sequences which direct expression of the microtrophin in a host cell.
2. The nucleic acid molecule according to claim 1, wherein the microtrophin comprises an internal deletion of the native utrophin protein of hinge region 3.
4. The nucleic acid molecule according to claim 1, wherein the microtrophin comprises a C-terminal deletion from exon 63 through the C-terminal amino acid of the native utrophin protein.
5. The nucleic acid molecule according to claim 1, wherein the microtrophin comprises the N-terminal sequences of utrophin through at least two hinge regions, and a C-terminal region from repeat 22 through exon 63.
6. The nucleic acid molecule according to claim 1, wherein the microtrophin is selected from the group consisting of human microtrophin having the amino acid sequence of SEQ ID NO: 4, canine microtrophin having the amino acid sequence of SEQ ID NO:2, and mouse microtrophin having the amino acid sequence of SEQ ID NO:5.
7. The nucleic acid molecule according to claim 1, wherein the regulatory sequences comprise a constitutive promoter.
8. The nucleic acid molecule according to claim 1, wherein the regulatory sequences comprise a muscle-specific promoter.
9. A vector comprising the nucleic acid molecule of any of claims 1 to 8.

10. The vector according to claim 9, wherein said vector is selected from the group consisting of an adeno-associated viral vector and a plasmid vector.

11. A pharmaceutical composition comprising a vector according to claim 9 or 10 and a physiologically compatible carrier.

12. The pharmaceutical composition according to claim 11, wherein the carrier is a buffered saline solution.

13. Use of a nucleic acid molecule according to any of claims 1 – 8 in preparing a medicament.

14. Use according to claim 13 wherein the medicament is useful for treatment of muscular disorders.

15. Use according to claim 13 wherein the medicament is useful for treatment of Duchenne Muscular Dystrophy.

16. A method of treating dystrophin deficiency by delivery of a vector comprising a nucleic acid molecule according to claim 1 and a physiologically compatible carrier.

17. The method according to claim 16, wherein the vector is an adeno-associated viral vector.